

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau(43) International Publication Date
17 July 2003 (17.07.2003)

PCT

(10) International Publication Number
WO 03/057179 A2

- (51) International Patent Classification⁷: **A61K**
- (21) International Application Number: **PCT/US03/00894**
- (22) International Filing Date: 10 January 2003 (10.01.2003)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data:
60/347,758 11 January 2002 (11.01.2002) US
- (71) Applicant (*for all designated States except US*):
BIOMARIN PHARMACEUTICAL, INC. [US/US];
371 Bel Marin Keys Blvd., Suite 210, Novato, CA 94949 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (*for US only*): **STARR, Christopher, M.** [US/US]; 19535 Seventh Street East, Sonoma, CA 95476 (US). **ZANKEL, Todd** [US/US]; 46 Galli Drive, Novato, CA 94949 (US).
- (74) Agents: **MYCROFT, Frank, J.** et al.; Townsend and Townsend and Crew LLP, Two Embarcadero Center, 8th Floor, San Francisco, CA 94111-3834 (US).
- (81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (*regional*): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- Declaration under Rule 4.17:**
— of inventorship (Rule 4.17(iv)) for US only
- Published:**
— without international search report and to be republished upon receipt of that report
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.*

(54) Title: USE OF P97 AS AN ENZYME DELIVERY SYSTEM FOR THE DELIVERY OF THERAPEUTIC LYSOSOMAL ENZYMES



(57) Abstract: The present invention provides for compositions and methods for treating, ameliorating or preventing a lysosomal storage disease by administering to a patient suffering from a lysosomal storage disease a P97 conjugated with an enzyme which is capable of transportation into the lysosomes of cells on either sides of the blood brain barrier.